SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

MAY 2016

Chairwoman Katherine S. Kazarian

Report Submitted to the Rhode Island House of Representatives

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I. Commission Members

· Representative Katherine S. Kazarian (D-Dist. 63, East Providence) - Chairwoman

· Representative David A. Bennett (D-Dist. 20, Warwick, Cranston)

· Representative Michael W. Chippendale (R-Dist. 40, Foster, Coventry, Gloucester)

· Dr. Ailis Clyne, Medical Director of the Rhode Island Department of Health Division of Community, Family Health and Equity

· Mr. Michael Souza, Executive Director of the Hospital Association of Rhode Island

· Dr. Terri Fox Wetle, Dean of the Brown University School of Public Health

· Ms. Ginny Law, a member of the public with a rare disease

· Ms. Patricia Weltin, a caregiver of an individual with a rare disease

- Ms. Kara Butler, Rhode Island Public Health Association

II. Letter from Chairwoman Kazarian

State of Rhode Island and Providence Plantations

HOUSE OF REPRESENTATIVES

REPRESENTATIVE KATHERINE S. KAZARIAN *District 63*

Committee on Corporations

Committee on Rules

A SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

I am pleased to submit this report of the commission's findings to the House of Representatives. I would like to sincerely thank every member of the commission for their hard work and dedication; Rep. David A. Bennett, Representative Michael W. Chippendale, Dr. Ailis Clyne, Mr. Michael Souza, Ms. Kara Butler, Dr. Terri Fox Wetle, Ms. Ginny Law and Ms. Patricia Weltin. I would also like to thank Kendra Cervone and CJ Donovan of the House Policy Office for administering the commission as well as their tireless efforts in the research, interpretation, and overall compilation of both printed and digital information that the commission received throughout its efforts.

Over the past six months the commission has received and compiled a great deal of information from many of the stakeholders associated with the issue of Rare Disease. The commission was able to establish a better understanding of processes and practices associated from those suffering as well as their caretakers. This report outlines the findings and makes recommendations by identifying areas of weakness and strength.

With the efforts of the insurance industry, pharmacy industry, physicians, caretakers and those suffering from rare disease it is my sincere hope that this report will result in an improved coordination of resources for providing care to those afflicted individuals. I am further hopeful that one of the most important aspects of this report and its findings will be a better understanding and communication between all parties affected by this unfortunate dilemma facing the State of Rhode Island.

Respectfully submitted,

Chairwoman Katherine S. Kazarian

380 PLEASANT STREET
EAST PROVIDENCE, RHODE ISLAND 02916

III. House Resolution Creating Study Commission 2015 –H 5297 Substitute A

2015 -- H 5297 SUBSTITUTE A
======== LC000901/SUB A ========
STATE OF RHODE ISLAND
IN GENERAL ASSEMBLY
JANUARY SESSION, A.D. 2015

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H O U S E R E S O L U T I O N
CREATING A SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

Introduced By: Representatives Kazarian, and Amore

Date Introduced: February 04, 2015

Referred To: House Health, Education & Welfare

RESOLVED, That a special legislative commission be and the same is hereby created consisting of nine (9) members: three (3) of whom shall be members of the Rhode Island House of Representatives, not more than two (2) from the same political party, to be appointed by the Speaker of the House; one of whom shall be the Director of the Rhode Island Department of Health, or designee; one of whom shall be the Executive Director of the Rhode Island Hospital Association, or designee; one of whom shall be an individual diagnosed with a rare disease, to be appointed by the Speaker of the House; one of whom shall be a caregiver of an individual diagnosed with a rare disease, to be appointed by the Speaker of the House; one of whom shall be the Associate Dean of Medicine for Public Health and Policy at the Alpert Medical School at Brown University, or designee; and one of whom shall be the President of the Rhode Island Public Health Association, or designee.

In lieu of any appointment of a member of the legislature to a permanent advisory commission, a legislative study commission, or any commission created by a General Assembly resolution, the appointing authority may appoint a member of the general public to serve in lieu of a legislator,

provided that the Majority Leader or the Minority Leader of the political party which is entitled to the appointment, consents to the member of the general public. The purpose of said commission shall be to make a comprehensive study and offer recommendations for coordinating the efforts of state resources, private entities, and social services in order to efficiently provide care for Rhode Islanders living with rare diseases and their caregivers. Forthwith upon passage of this resolution, the members of the commission shall meet at the call of the Speaker of the House and organize and shall select a chairperson. Vacancies in said commission shall be filled in like manner as the original appointment. The membership of said commission shall receive no compensation for their services. All departments and agencies of the state shall furnish such advice and information, documentary and otherwise, to said commission and its agents as is deemed necessary or desirable by the commission to facilitate the purposes of this resolution.

The Speaker of the House is hereby authorized and directed to provide suitable quarters for said commission; and be it further

RESOLVED, that the commission shall report its findings and recommendations to the House of Representatives no later than March 24, 2016, and said commission shall expire on June 24, 2016.

EXPLANATION BY THE LEGISLATIVE COUNCIL OF
H O U S E R E S O L U T I O N
CREATING A SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

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This resolution would create a nine (9) member special legislative study commission whose purpose it would be to study and make recommendations for coordinating the necessary resources to provide care to individuals with rare diseases, and who would report back to the House no later than March 24, 2016, and whose life would expire on June 24, 2016.

IV. **Executive Summary**

The Rhode Island House of Representatives created the Special Legislative Study Commission to Coordinate Resources for Providing Care to Individuals with Rare Diseases. The commission was created by H 5297 SUBSTITUTE A.

Over the course of several months, the commission heard presentations from individuals who currently have rare diseases, their caregivers, two Rhode Island health insurance companies (Blue Cross Blue Shield of Rhode Island and Neighborhood Health Plan of Rhode Island), The Rhode Island Department of Health, Care New England, and various physicians, among others.

The commission sought to determine more effective ways of coordinating current resources to address the underserved needs of individuals with rare diseases. It is the hope that a commission of this nature will not only further help and assist the many affected rare disease patients in Rhode Island but also serve as a catalyst to spur economic activity in the state. This is a unique opportunity for the state to partner with many stakeholders; utilizing resources currently available in anticipation of those that, going forward, will soon be available as the knowledge district begins to unfold.

Creating a unique collaborative partnership between patients, providers, scientists, agencies and institutions of learning will allow the State of Rhode Island to be a leader and role model for other states to follow.

V. Commission Recommendations

The commission recommends that the House of Representatives extend and expands this existing commission to continue the collaboration between physicians, caregivers, individuals with rare diseases, the health insurance industry and other stakeholders. Below is further information on this commission.

Proposed Rhode Island Rare Disease Commission	• That the composition of said commission is hereby amended to consist of twenty (20) members: three (3) of whom shall be members of the Rhode Island House of Representatives, not more than two (2) from the same political party, to be appointed by the Speaker of the House; one of whom shall be the Director of the Rhode Island Department of Health, or designee; one of whom shall be the Executive Director of the Rhode Island Hospital Association, or designee; two (2) of whom shall be individuals diagnosed with a rare disease, to be appointed by the Speaker of the House; one of whom shall be a caregiver of an individual diagnosed with a rare disease, to be appointed by the Speaker of the House; one of whom shall be the Associate Dean of Medicine for Public Health and Policy at the Alpert Medical School at Brown University, or designee; one of whom shall be the President of the Rhode Island Public Health Association, or designee; three (3) of whom shall be representatives of Health Insurance Companies, to be appointed by the Speaker of the House; two (2) of whom shall be representatives of the Pharmaceutical Industry, to be appointed by the Speaker of the House; one of whom shall be a representative of the biotech industry, to be appointed by the Speaker of the House; three (3) of whom shall be physicians, to be appointed by the Speaker of the House; and one of whom shall be a representative from a rare disease organization, to be appointed by the Speaker of the House; and be it further.
Terms and Length of Commission	• Said commission would expire on June 24, 2019 • The Commission is required to meet at least (3) times in the calendar year.
Required Recommendations	• Examine research, diagnoses, treatment and education relating to rare diseases and • Examine benefits of center of excellence approach with the providers.
Responsibilities	• This resolution would expand the House Special Legislative Study Commission to (20) members and whose purpose it would be to study and make recommendations for coordinating the necessary resources to provide care to individuals with rare diseases.

VI. Summary of Notes of Commission Meetings

October 27, 2015 Meeting

H O U S E R E S O L U T I O N
CREATING A SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

Summary Meeting Notes

Meeting called to order by Deputy Chief of Staff John Conti, on a motion from Representative David Bennett, Rep Katharine Kazarian was elected Chairwoman. Representative Bennett was elected Vice-Chair and Representative Chippendale as Secretary.

Chairwoman Kazarian welcomed everyone, and gave a background description of the commission and its duties. A rare disease is defined as a disorder that affects fewer than 200,000 people at any given time, presently there are 7,000 rare diseases affecting 30 million Americans. (1 in every 10 Americans)

Many of those suffering often have issues with their diagnosis; statistics show that on average it takes 8 years to be properly diagnosed. The rarity of a person's disease makes medical research more difficult.

The intent of this commission is to better understand the challenges individuals with rare diseases and their caretakers face in order to find more effective practices to be utilized as a state.

Brief background and introduction of commission members:

Representative David Bennett is a registered nurse who is concerned with this important subject matter.

Representative Michael Chippendale informed the commission that he is uniquely positioned, as a person with 3 rare diseases himself. He understands firsthand the issues facing those suffering with rare disease.

Dr. Ailis Clyne was a pediatrician for 16 years and also personally affected with a child who fits the definition of rare disease.

Michael Souza explained his desire to learn and help with the coordinated resources aspect.

Ginny Law, who has rare diseases, told the members that it took 50 years to diagnose her situation and would like to see others spared that path through this commission's work.

Terrie Fox Wetle spoke of the concern with population health, and a deep understanding of "rare diseases" through her work at Brown University regarding Progeria.

Ms. Patricia Weltin is the CEO of Rare Disease United Foundation; she is looking to help with the lack of understanding surrounding the issues of rare disease, in addition to the significant savings that could be realized by addressing this subject matter in a proactive manner. Currently the cost to the country is roughly $500 billion dollars a year. She is a caregiver for her children, who have multiple rare diseases.

Representative Kazarian concluded the meeting by expressing her desire to invite all the different stake holders to future meetings to help learn what we can do as a state for this community.

November 17, 2015 Meeting

H O U S E R E S O L U T I O N
CREATING A SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

Summary Meeting Notes

Chairwoman Kazarian welcomed everyone and went over the slate of agenda items that would take place at the meeting.

Presentation by Dr. Ailis Clyne, medical director
at the Rhode Island Department of Health

Chairwoman Kazarian invited the first speaker/presenter, Dr. Ailis Clyne, a doctor and medical director at the Rhode Island Department of Health. She explained that her presentation would highlight related activities and programs that currently go on at the Department of Health that are related to helping and supporting individuals with Rare Diseases. She began by defining a rare disease, which is a disease that affects fewer than 200,000 Americans at any given time. She explained that there are currently 6,000 rare diseases. She also highlighted the fact that 80% of Rare Diseases have identified genetic origins. She explained that the Department of Health provides resources to individuals with rare diseases through their Office of Special Needs, Newborn Screening Program, and the Birth Defects Program.

The Office of Special Needs is funded by Title V Block Grant-funded maternal child health. They provide programs on:

- Disability and Health
- Pediatric Practice Enhancement Project
- Adolescent transition to adult healthcare
- Family Peer Resource Specialist Program
- Special Needs Peer Resource Specialist Program

The Disability and Health Program ensures that people with disabilities have access to all public health and health promotion programs, such as preventive health screening, emergency preparedness, and access to healthcare facilities.

The program promotes the health and wellness of people with disabilities through training, technical assistance, and evidence-based, self-management programs.

Disability and Health Program works with state and community-based partners to reduce health disparities due to disability status.

Dr. Clyne explained that the goals of Newborn Screening are as follows:

1. All infants receive newborn bloodspot screen
2. All infants with abnormal result(s) receive follow-up
3. Healthcare providers and families receive information on screening results, implications for child's health, recommendations for follow-up/evaluation/treatment
4. All infants with confirmed disorder receive appropriate treatment
5. All individuals with confirmed disorder will have continuous access to treatment in order to optimize health and developmental outcomes.

Regarding The Birth Defects Program focused on structural abnormalities that affect the development of organs and tissues of an infant or child.

The Birth Defects Program does the following:

- Identify children (birth – age 5) with birth defects and monitor prevalence trends
- Develop effective strategies for primary and secondary prevention of birth defects
- Assure children with birth defects and their families are linked to and receive appropriate services on a timely basis
- Assure access to information for all regarding prevalence, prevention, and services

Testimony by individuals with Rare Diseases and
Caregivers of people with Rare Diseases

Tiffany Marie Adams explained to the commission members that she has a rare disease called Chiari Malformation. She explained that this has impacted her life greatly. She was diagnosed following a car accident. She has terrible headaches, neck pain, gets numbness in her legs, and autonomic impairments, nervous system and central nervous systems, and parasympathetic nervous system issues. She explained that she is having a problem finding true specialists in the state of Rhode Island for this condition. She said that her insurance is with Neighborhood Health Plan. However, there are only 16 specialists for her condition in the country. She was able to find a doctor in North Carolina. She had to raise money for herself in order to pay for all of medical expenses and travel associated with managing her condition. She explained that the #1 cause of death of people with Chiari Malformation is sleep apnea. She currently runs a support group which has 64 members. She feels as though the State of Rhode Island would save money if there was better coordination between providers.

The next speaker was Dorie Carter with her son, Eric Carter. Dorie is a caregiver to her son, Eric, who has a rare disease. He has complex medical and developmental needs. At age 5, he was diagnosed with a very rare metabolic disorder called NKH. He is said to be the only child with this disorder in Rhode Island. She struggles with finding a pharmacy that can create his unique complex of medications and their insurance does not cover his medication. She also struggles at the school level in garnering the appropriate accommodations for her son due to his unique needs. She explained further that her health has suffered a great deal due to the high degree of stress levels that she has due to her son's condition and lack of resources and help they are receiving.

The next presenter was Patricia Hanley. She explained that she suffers from generalized dystonia. This disorder causes random muscles to contrast, twist, and stiffen into awkward postures. It is very painful and debilitating. She explained that she depends on the MBTA to get to her doctor's appointments in Boston. They came to the conclusion that the only way she could get to her appointments was indeed the MBTA. However, the winter of 2014 was very bad with many storms. This made her condition worse, since dystonia becomes worse in the colder weather. At this point she has Neighborhood Health Insurance and everyone with Neighborhood Health has a case manager. It has a program that helps low income disabled people get to their doctor's appointment. She now receives rides to her doctor's appointments.

Nicole Dreyer-Gavin and her son Ian explained that diagnosed with PKU through newborn screening. They received care at Rhode Island Hospital. For the first few years, he received all of the necessary medical food and formula from the hospital. After this, things changed, where they had to go through insurance in order to obtain this food, which Ian needs to survive. She explained that she and Ian have lobbied in Washington DC for food coverage. The out of pocket costs are approximately $25,000 a year and quite expensive.

Next was Stuart Smith. He explained that he is caregiver to his wife, who has a rare disease. He told the commission that his wife is not present today because she is home recovering from her 23rd surgery. He explained that his wife had to give up her profession due to her condition. Due to the lack of resources that exist for individuals with rare diseases, it is oftentimes left in the hands of caregivers.

December 9, 2015 Meeting

H O U S E R E S O L U T I O N
CREATING A SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

Summary Meeting Notes

Chairwoman Kazarian opened the meeting with Brenda McGovern, Director of Case Management for Blue Cross Blue Shield of Rhode Island. A background on how BCBSRI integrates and works with their members who have rare conditions was presented to the commission.

According to Brenda McGovern a complex case management model is utilized. Nurses are used as a resource and quarterback in identifying and aiding those willing to participate. The process then begins by helping patients identify the gaps and barriers associated with their care. Issues like education and resources are analyzed in conjunction with a patient's physician. A team like atmosphere is fostered by including dieticians, social workers and the behavioral health care component of individuals for as long as needed. The Johns Hopkins method is utilized by Blue Cross Blue Shield of Rhode Island. Patricia Weltin asked about the process and if it could be more efficient. She feels more coordinated effort should be utilized and sees a potential for significant savings in doing so.

Ginny Law noted BCBSRI's involvement with her rare disease and inquired about the best way to help case mangers better understand what a patient or caregiver already knows about their condition. Testimony suggested that all parties could benefit through a working partnership. Dr. Clyne inquired about access to centers of excellence and telemedicine programs, both of which BCBSRI said they participate in.

Representative Chippendale would like to see some type of a central repository for a patient to translate information. Possibly a rare disease hotline could be added onto the back of provider ID cards. This could help add a level of confidence for patients in addition to assigned case managers. Dr. Francisco Trilla, Chief Medical Officer at Neighborhood Health Plan RI. stated the best way to address the issue of rare disease is to coordinate communicate and continue to improve. Rare disease can be very complex and challenging to all parties including resources on the state. All stakeholders stand to benefit with an ongoing dialogue, he used as an example the issue of AID's which at one point was considered a "rare disease". Trying to balance coordinated resources against wasted resources is the key to success in this area. Patty Weltin suggested advantages and value with investing in programs that would help reduce diagnosis times. RI has the ability to be the leader in paving the way for rare diseases.

Dr. Clyne stressed the benefits associated with a consistency of doctor's and coordination of efforts across all 3rd party payers to harmonize care efforts so as not to disrupt those who have good coordinated care in place. Both Brenda McGovern and Dr. Trilla both agreed that this effort would put in place a system of centralization allowing case managers who are stuck to see if the task force has something to add or additional resources to offer.

Representative Kazarian concluded the meeting by extending gratitude, on behalf of the members, to BCBSRI and Neighborhood Health Plan RI. The first step is starting the conversation and that's what has been accomplished here today according to Chairwoman Kazarian.

March 2, 2016 Meeting

H O U S E R E S O L U T I O N
CREATING A SPECIAL LEGISLATIVE COMMISSION TO STUDY THE CREATION OF A COUNCIL TO COORDINATE RESOURCES FOR PROVIDING CARE TO INDIVIDUALS WITH RARE DISEASES

Summary Meeting Notes

Chairwoman Kazarian opened the commission's final meeting and expressed sincere disappointment with United Health Care's unwillingness to participate in this important discussion.

She introduced Dr. Legare of Care New England as the final speaker and asked for his input on the rare disease subject matter. Dr. Legare expressed the importance surrounding allocation of resources but stressed that it shouldn't be the only deciding factor for patients and treatment decisions. He advocated the importance of nurse navigators and just how indispensable they are in the process. They act as the glue for patients as they embark on a sometimes very complex journey through the system. This position is an advocate for the patient many times helping with informed medical decisions. He also expressed how imperative communication is for long term success by avoiding duplication and waste along the way.

Patricia Weltin described the role of caretaker as a full time job; unfortunately it feels like the rare disease segment of society is often less cared for and underserved than others. She would like to model a system after that of others such as those suffering with Cancer. Palliative care programs are extremely useful especially when used in conjunction with nurse navigators. Representative Chippendale expressed his frustration with the lack of coordination with information. He asked about the possibility of a "clearing house" and what possible tools are available for patients suffering from rare disease.

Dr. Legare certainly sees the need for improvement regarding database and the sharing of information. Patricia Weltin asked Dr. Legare if he saw

the benefit in a task force or advisory council in trying to coordinate efforts for those suffering. Dr. Legare agreed with this type of advocacy and educational opportunities that it would provide. He expressed that all parties do seem to be on the same page but communication among all stakeholders is key to getting there.

Ginny Law added the school issues for children as an important aspect of the discussion. It needs to be addressed on a certain level so that those affected understand the ramifications extend beyond the hospitals and treatment facilities into the everyday lives of those suffering.

Dr. Fox Wetle is very supportive of the goals defined in the legislation, but has concerns that this may become an unfunded mandate because of the limited resources. She does not want to see these very important and admirable goals get lost due to a lack of sufficient resources. Dr. Clyne also agrees with the high level goals of the commission but expressed caution on forming this entity as the method of getting there.

Chairwoman Kazarian stated after serving on this commission and listening to discussion that something needs to be done. All presenters to the commission agreed that this would provide those suffering a platform to dialogue with the various stakeholders. Informed and better communication was a common thread used throughout all of the testimony.